Successful SCID gene therapy in infant with disseminated BCG.

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Public Summary:
Treatment options for patients with SCID beyond standard matched sibling donor transplant are not ideal and come with the risk of transplant related morbidity. These complications become particularly worrisome in patients with active infections. Hence, alternative therapies limiting conditioning regimen yet allowing full reconstitution are needed. Herein, we report a rare case of an infant with XSCID and disseminated BCG infection, who was successfully treated on our gene therapy trial combining low dose busulfan with the infusion of lentiviral-transduced, gene-corrected autologous stem cells. The patient required multidrug antimycobacterial therapy that was continued during busulfan conditioning without any side effects including hepatotoxicity. Full functional immune reconstitution and MAT resulted in complete resolution of BCG infection without development of immune reconstitution syndrome. Thus, gene therapy with low dose busulfan might be a promising treatment option for patients with life threatening infections.

Scientific Abstract: